

## Summary of the RCRP Stakeholders Meeting

The RCRP held a virtual Stakeholders meeting in April 15<sup>th</sup>, 2020 that was attended by 37 Stakeholders from 22 organizations/institutes in the field, including clinicians, researchers, patient advocacy groups, and representatives from FDA and NIH. Attendees were invited to share broad perspectives on what initiatives are needed to propel the rare cancers field forward and break down potential barriers. Emphasis was placed on how to define and distinguish the RCRP from other CMDRP cancer programs, strategies for improving collaboration and data sharing, and the development of an approach to address issues common across rare cancers.

During the Stakeholders meeting, participants were asked three critical questions, which will help setting up goals, identifying focus, and developing strategic planning for the RCRP.

A summarized list of the responses to each question is provided below.

### **Question #1: How should we define rare cancers from a research perspective?**

- Those cancers affecting < 6 persons per 100,000 per year in the U.S.
- Emphasis on those rare cancers without approved targeted/effective therapies or adequate treatment guidelines; those with the highest prevalence, morbidity, and mortality; and those lacking appropriate research tools/models.
- FDA definition: rare disease that affects less than 200,000 persons in the US
- Molecular classification

### **Question #2: What should the RCRP consider to improve collaboration and data sharing?**

- Generate databases/banks for centralizing and sharing data, with a common data structure which PIs are required to participate in as a condition of funding.
- Compile registries for reagents, models, and patients including a centralized rare tumor bio-specimen repository with clinical annotation.
- Encourage partnership with patient advocacy organizations.
- Encourage international studies.
- Consortia model or multi-site/investigator collaborative projects

### **Question #3: What do you see as the key features of a “platform approach” to addressing common issues of rare cancer research?**

#### **Research Priorities:**

- Development and validation of pre-clinical models, including tumor and animal models, which may be applicable across multiple rare cancers or common cancer types and can support clinical trial readiness.
- Identifying shared etiologies, molecular pathways and tissue specific features (microenvironmental factors)
- Novel combination therapies and drug repurposing
- Longitudinal studies of disease natural history and treatment response
- Research based on small sample sizes

#### **Other Approaches:**

- Focus on beginning of the pipeline, idea/concept awards that can initiate a larger study (Use the "stick" of future funding)
- Basket trials
- Promoting new investigator